



A tool for rapid development of clinical-grade protocols for dopaminergic neuronal differentiation of Parkinson's Disease patient-derived iPSCs

Grant Award Details

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Grant Type: Quest - Discovery Stage Research Projects

Grant Number: DISC2-10067

Project Objective: Developing a tool for rapid development of clinical-grade protocols for dopaminergic neuronal

differentiation of Parkinson's Disease patient-derived iPSCs

Investigator:

Name: Justin Cooper-White

Institution: Scaled Biolabs Inc.

Type: PI

Disease Focus: Parkinson's Disease, Neurological Disorders

Award Value: \$657,528

Status: Active

Grant Application Details

Application Title: A tool for rapid development of clinical-grade protocols for dopaminergic neuronal differentiation

of Parkinson's Disease patient-derived iPSCs

Public Abstract:

Research Objective

Develop a tool that facilitates rapid, cost effective development of optimized GMP-grade hPSC differentiation into functional DA neurons and apply this device to a cohort of PD patient-derived iPSCs.

Impact

Creating GMP-grade, functionally consistent phenotypes for DA neurons from each patient will significantly increase the likelihood of stem cell-derived DA neuron-based therapy for PD sufferers.

Major Proposed Activities

- Develop a microfluidic device platform based on Scaled Biolabs methodology and approach that enables high-throughput optimization of a multistage, multifactor differentiation protocol.
- Validate these new tools with the gold standard WA09 hESC line and the current best performing patient iPSC line using our research-grade DA neuronal differentiation protocol.
- Transition research-grade protocol to GMP-grade protocol with gold standard WA09 hESC line and the current best performing patient iPSC line
- Implement the tool to achieve optimized GMP-grade differentiation conditions for the generation of phenotypically and functionally equivalent DA neurons from eight PD patient
- Translate optimized GMP-grade differentiation conditions for each cell line to larger scale tissue culture plate/flask-based cultures and characterize using genomic analysis and electrophysiology.

California:

Statement of Benefit to Thousands of Californians suffer from the degenerative effects of Parkinson's disease, a disease for which there is no cure. Our study seeks to develop a tool to accelerate the clinical assessment of a possible solution for PD sufferers, the production of neurons that can be used treat PD patients with cells derived from their own stem cells. The same approach may be applied to other diseases, such as diabetes and heart disease, to the benefit of many of the citizens of California.

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